1.0 OBJECTIVES

1.1 Primary Aims

1.1.1 To evaluate the safety and toxicity of infusions of ex vivo expanded, gene marked donor bone marrow stromal cells following bone marrow transplantation in patients with some form of osteodysplasia.

1.2 Secondary Aims

1.2.1 To determine whether these ex vivo expanded, gene marked marrow stromal cells will engraft in the recipient's bone, bone marrow, and/or skin.

1.2.2 To begin to investigate whether high proliferative mesenchymal progenitor cells can be expanded ex vivo and retain their progenitor potential in vivo.

1.2.3 To begin to investigate whether ex vivo expanded bone marrow stromal cells can ameliorate the skeletal dysplasias associated with various genetic disorders.

2.0 BACKGROUND AND RATIONALE

Marrow stromal cells (MSC) contribute to the hematopoietic microenvironment and are capable of differentiating to osteoblasts and forming bone. This differentiation potential can be exploited in cell therapy (i) to enhance recovery of the marrow microenvironment after high dose chemotherapy and (ii) to enhance bone formation in the treatment of osteoporosis syndromes of children and adults. Although it has not been unequivocally demonstrated in humans, a key element for the success of this strategy is the capacity of MSCs to home to bone and bone marrow. This study is designed to test the hypothesis that allogeneic MSCs can be safely infused following bone marrow transplantation and will home to the recipient's bone and bone marrow.